Human Embryonic Stem Cell Therapeutic Strategies to Target HIV Disease

Grant Award Details

Human Embryonic Stem Cell Therapeutic Strategies to Target HIV Disease

Grant Type: Comprehensive Grant

Grant Number: RC1-00149

Project Objective: This project was designed to explore the potential for hematopoietic differentiation of human embryonic stem cells, and to then adapt these cells with genetic approaches to combat HIV infection of hematopoietic target cells derived from these hESC.

Investigator:

Name:	Zack Jerome
Institution:	University of California, Los Angeles
Type:	PI

Disease Focus: HIV/AIDS, Immune Disease, Infectious Disease

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $2,401,903

Status: Closed

Progress Reports

Reporting Period: Year 2

View Report

Reporting Period: Year 3

View Report

Reporting Period: Year 4

View Report

Reporting Period: NCE

View Report

Grant Application Details

Application Title: Human Embryonic Stem Cell Therapeutic Strategies to Target HIV Disease

Public Abstract: AIDS is a disease that currently has no cure. It arises when the human immunodeficiency virus (HIV) infects certain types of blood cells. These cells would normally be used to fight infection, but instead are destroyed by the virus, leading to immunodeficiency. We have recently been able to induce the development of human embryonic stem cells (hESC) into the types of cells that HIV can infect. In addition, we were able to show that a marker gene could be introduced into the hESC, and this gene continued to produce its protein throughout development of the cell into the more mature blood cell types. This sets the stage for testing the possibility of using gene-modified hESC to treat HIV or other immune system diseases. We have 3 different types of anti-HIV genetic approaches that we will test in laboratory models. These will be placed into hESC, and the cells allowed to develop into blood cells. We will then test whether our “therapeutic” genes can inhibit HIV infection in culture. We will also develop novel mouse models that allow development of hESC into blood cells in the body (in vivo). We will test the efficacy of certain of these genetic approaches in these systems, as they should more closely represent the situation in people. These studies will provide proof-of-principle that cells in the immune system can be modified by manipulation of hESC, and may help to develop future therapeutic approaches to combat HIV disease. In addition, these studies will be relevant to other immune system disorders such as autoimmune diseases.

It was estimated that by January 31 2005, approximately 151,000 Californians were HIV infected. Furthermore, according to the California HIV Surveillance Report, 1752 new cases of HIV infection (1700 adult and 52 pediatric cases), and 5 deaths were reported between April 1 and September 31, 2006. Current treatment strategies prolong life, but do not cure infection, and are themselves quite toxic. Consequently HIV disease, and improved therapeutic approaches for this disease, are issues of great importance to the people of California.

Statement of Benefit to California: Current treatment strategies to halt HIV infection (AIDS) prolong life, but do not cure infection, and are themselves quite toxic. There are over 150,000 Californians infected with the AIDS virus. Consequently HIV disease, and improved therapeutic approaches for this disease, are issues of great importance to the people of California. Our studies will explore the potential of using human embryonic stem cells to fight AIDS and HIV infection. We have shown that human embryonic stem cells can develope into the immune system cells that are destroyed by the AIDS virus. In addition, we are exploring ways to genetically modify these cells (gene therapy) so that they would be protected from infection, and be better able to fight the infection in the body. We hope to eventually use these genetically modified cells to treat HIV infected individuals. If successful, our results may allow HIV infected individuals to discontinue, or greatly reduce the amount of anti-viral drugs that they must now take. This could directly benefit the patients' health, cut the cost of therapy, and allow less productive time lost from work, thus benefitting the State economy as a whole.

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